Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease

Grant Award Details

Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease

Grant Type: Cure Sickle Cell Initiative Clinical Trial Stage Projects

Grant Number: CLIN2SCD-12031

Project Objective: Objective of the project is conduct a Phase 2 clinical trial of hematopoietic stem cell gene transfer inducing fetal hemoglobin in Sickle cell disease patients.

Investigator:

Name:	David Williams
Institution:	Boston Children's Hospital
Type:	PI

Disease Focus: Blood Disorders, Sickle Cell Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $8,333,581

Status: Pre-Active

Grant Application Details

Application Title: Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease

Public Abstract:

Therapeutic Candidate or Device

Autologous CD34+ cells transduced ex vivo with the BCH_BB694 LCR(-HS4) bGp D12shmiR lentiviral vector

Indication

Sickle cell disease with severe phenotype.

Therapeutic Mechanism

Silencing of beta-sickle globin and induction of anti-sickling fetal hemoglobin

Unmet Medical Need

Sickle cell is a severe disease with protean manifestations. The only curative therapy is an allogeneic stem cell transplant, optimally with an HLA-identical sibling donor. However, <20% of sickle cell patients have such a donor available.

Project Objective

Completion of pilot and instigation of phase 2

Major Proposed Activities

- Enrollment of one patient to fill remaining slot of pilot study and enrollment of patients on continuing phase 2 study.
- Development and validation of cell manufacturing in CA site to support west coast enrollment, including CA sites.
- Clinical and cell manufacturing site monitoring of CA sites.

Statement of Benefit to California:

Sickle cell disease (SCD) is a genetic condition mainly affecting African-Americans and those of certain Hispanic ethnic background. SCD has major morbidity with significant ongoing medical care costs. We are seeking a permanent therapy that mitigates the major morbidities. If successful, this treatment would significantly enhance the lives of affected individuals and reduce the lifelong financial burden of medical care, lost productivity and underemployment for individuals with severe SCD.
